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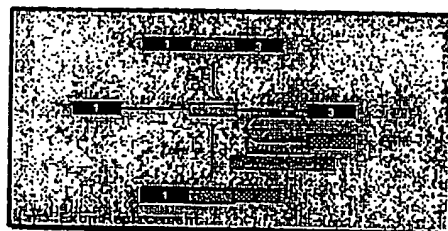
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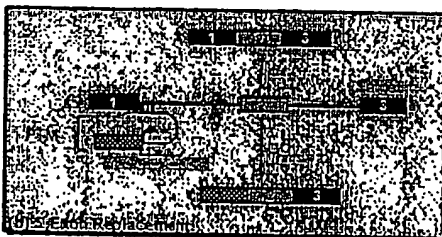


## FIGURE 1

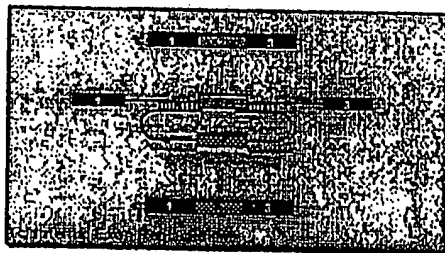
### Different SMaRT Reactions



3' ER



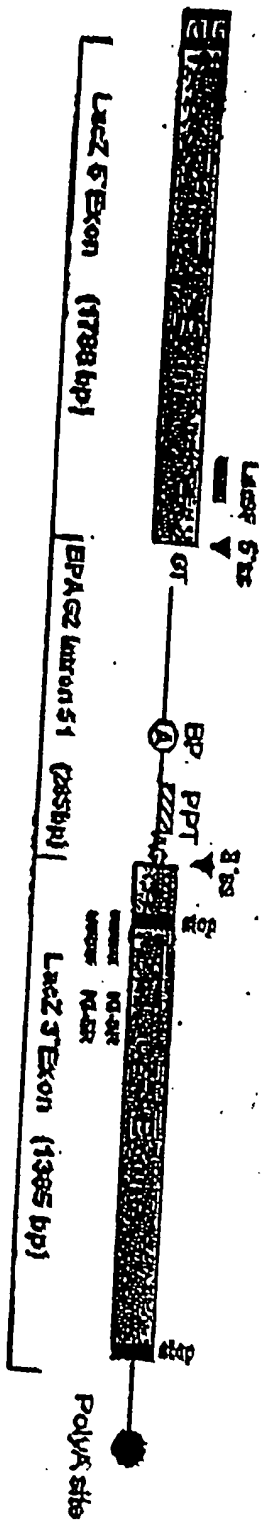
5' ER



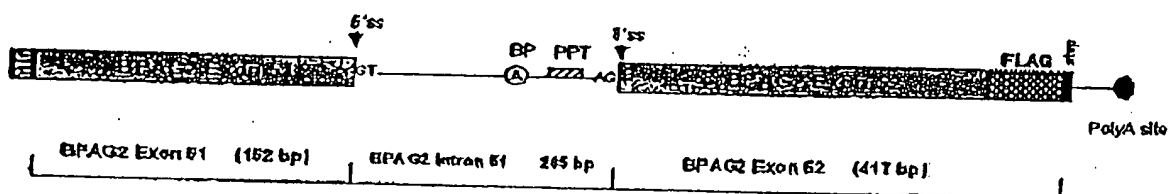
Internal

# 1) BPAG2 Target 1 (LacZ-T1)

## FIGURE 2A



2) BPAG2 Target 2 (T2)



**FIGURE 2B**

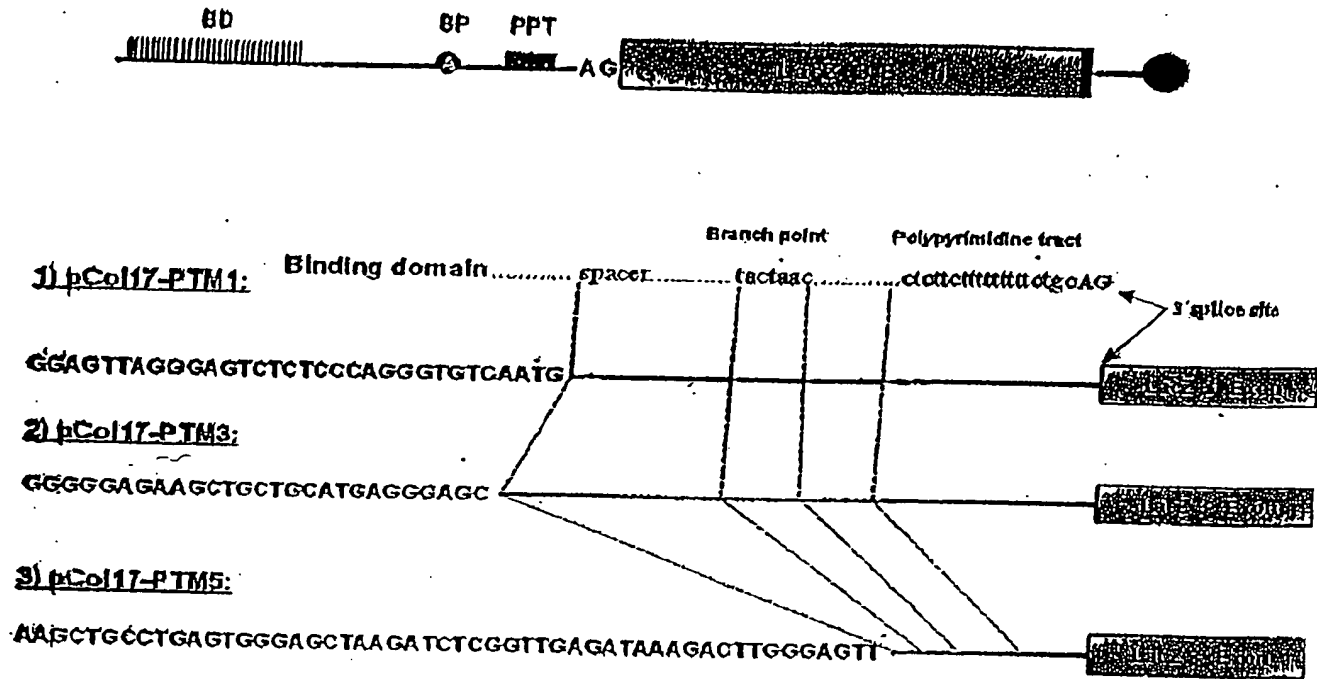


FIGURE 2C

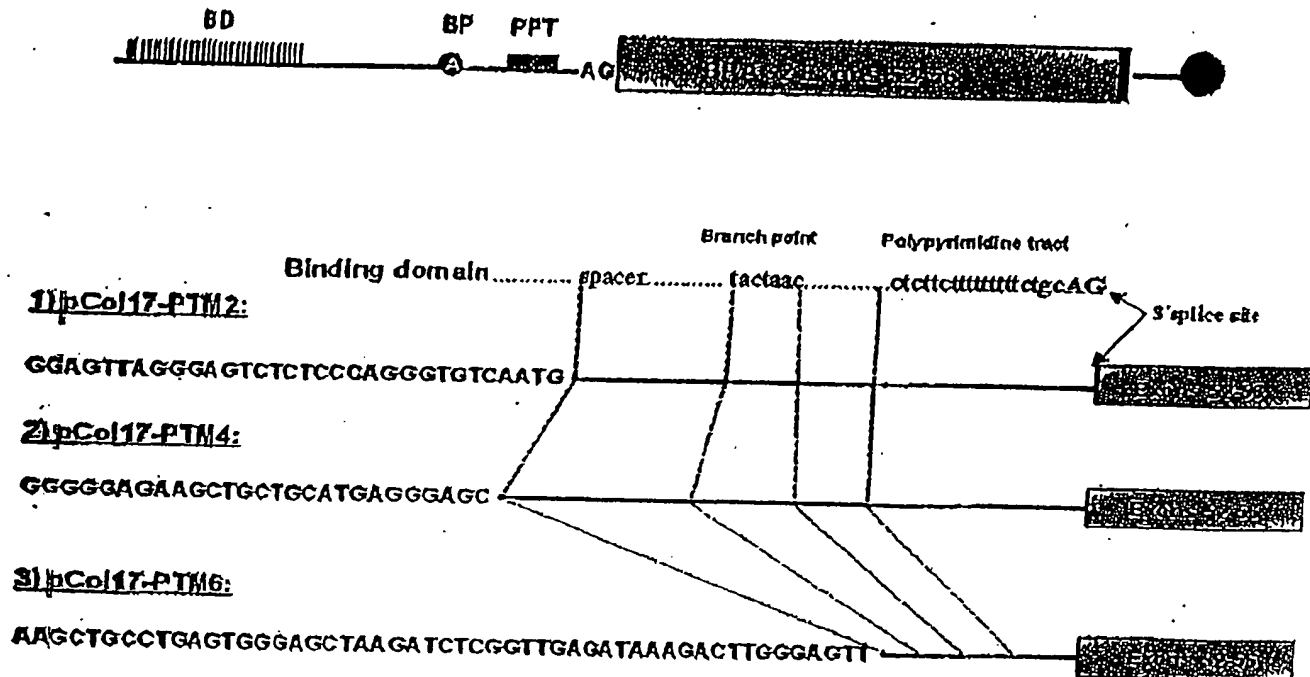
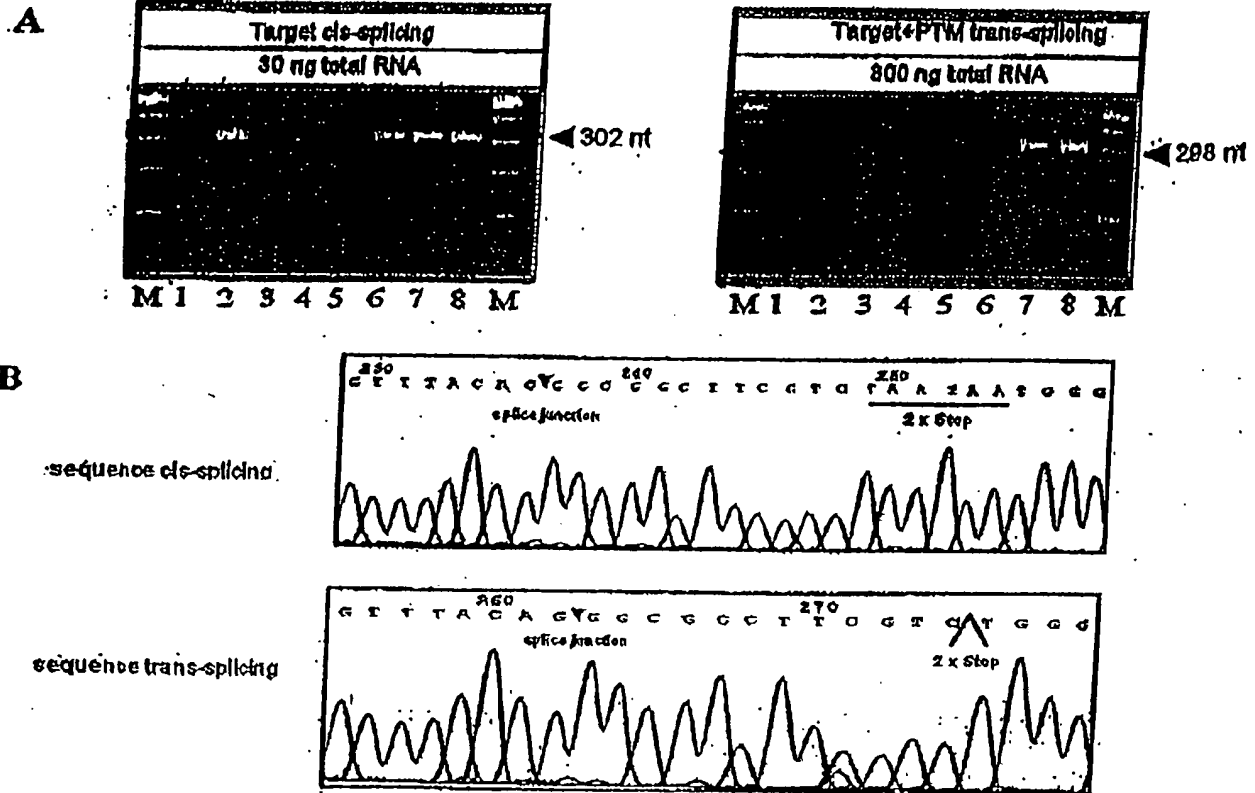


FIGURE 2D



**FIGURE 3A-B**

Q

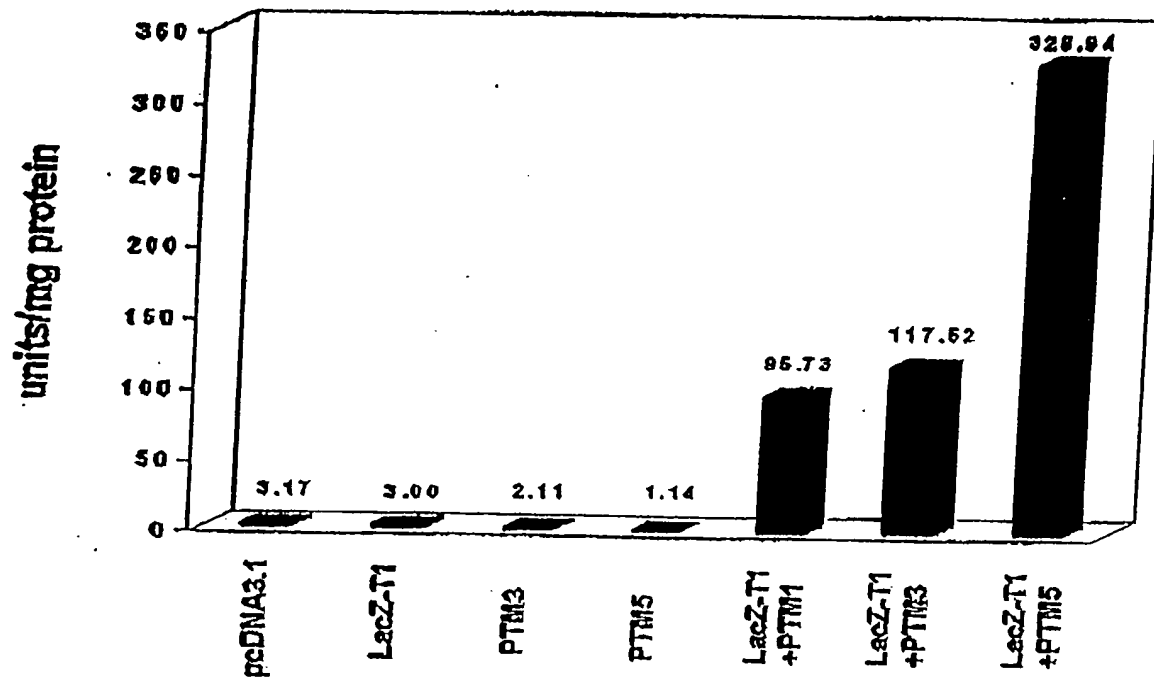
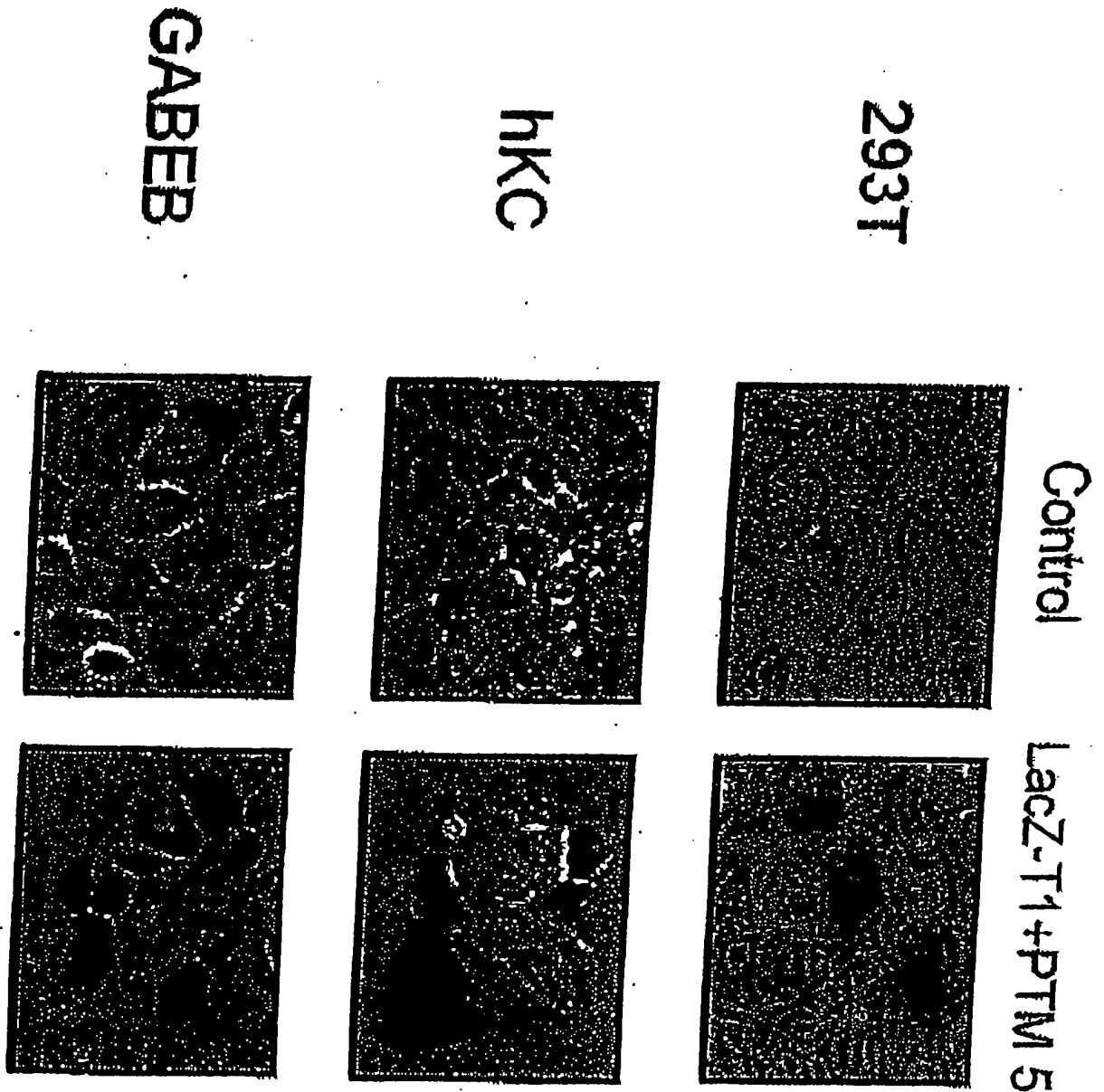


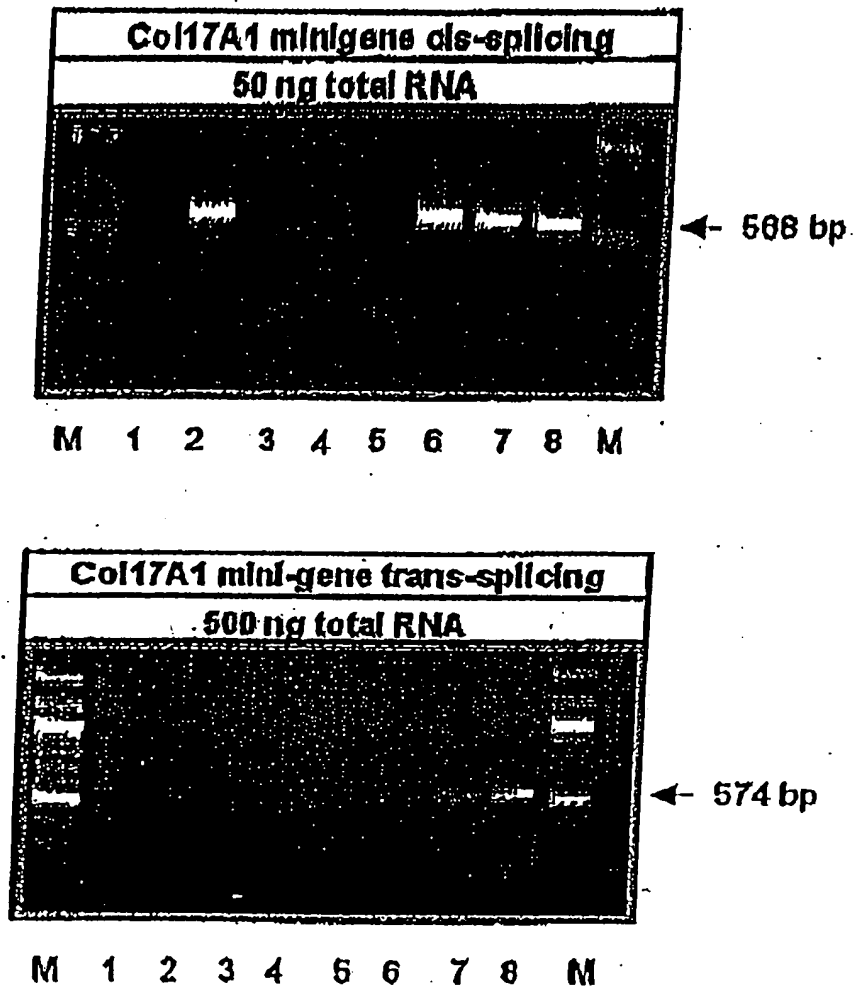
FIGURE 3C



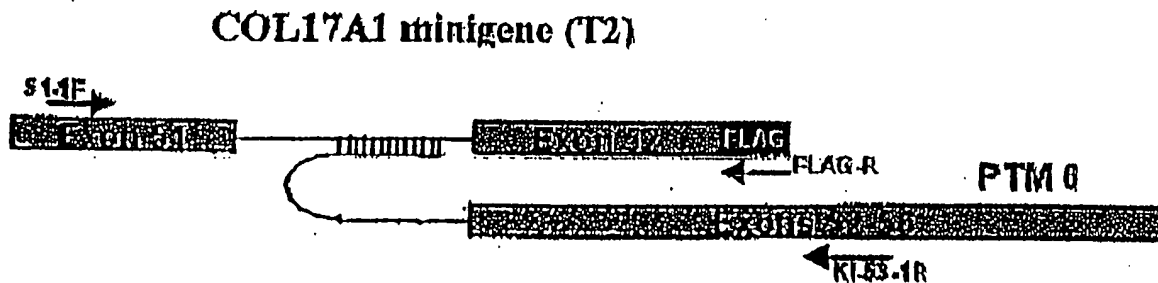


**FIGURE 4**

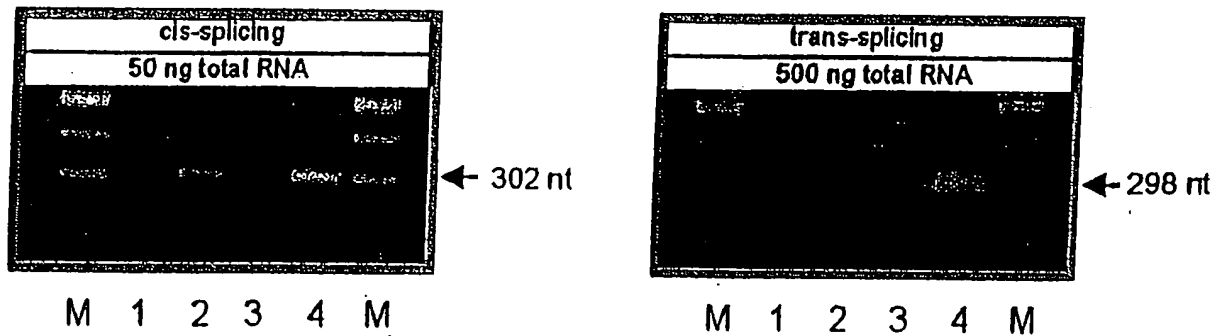
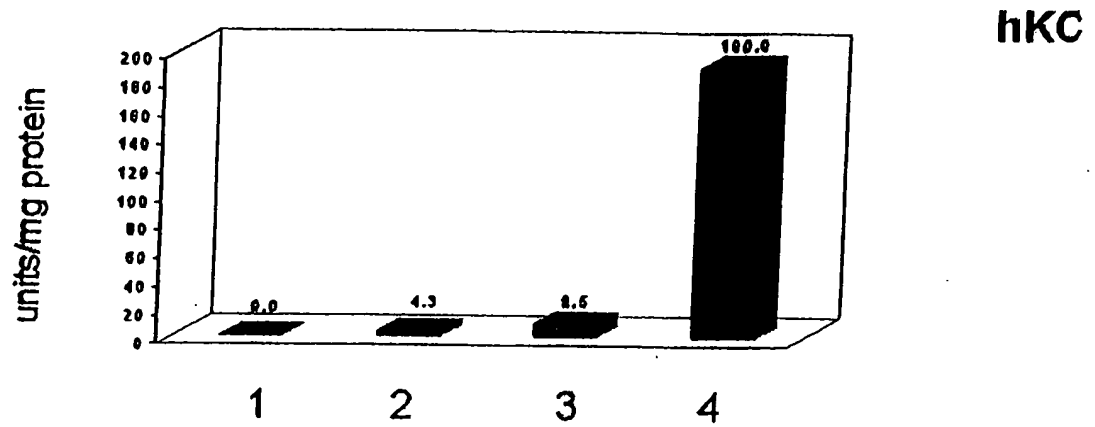
**A**



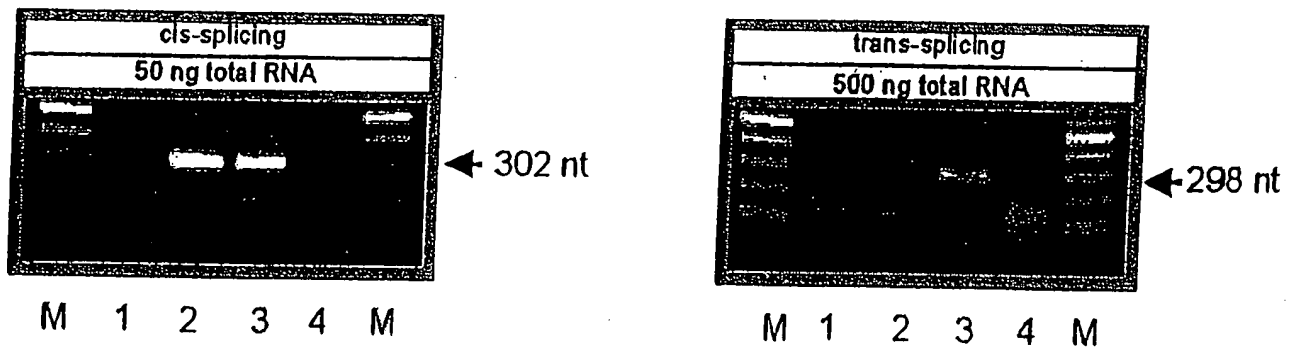
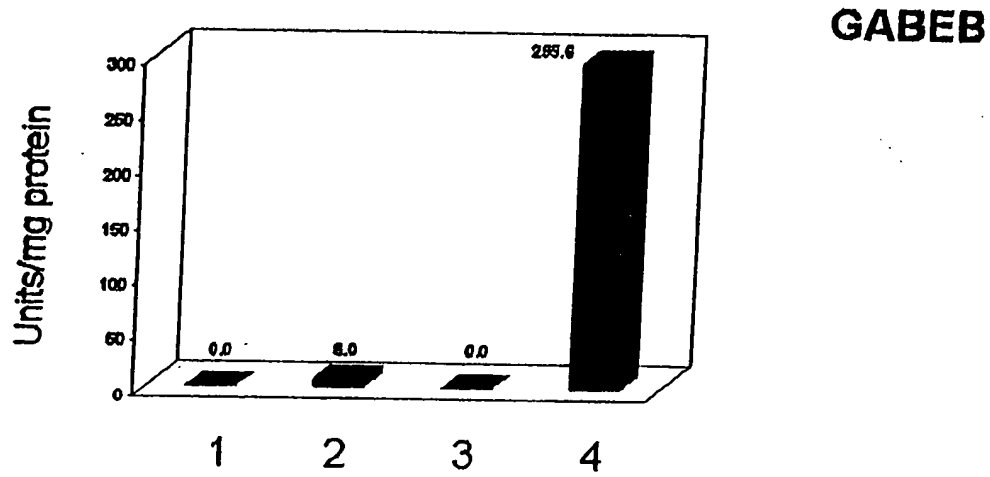
**B**



**FIGURE 5A-B**



**FIGURE 6A**



**FIGURE 6B**

**FIGURE 7**

**Cis-spliced mRNA**

**Col17A1 pre-mRNA**

**PTM 5 pre-mRNA**

**Trans-spliced mRNA**

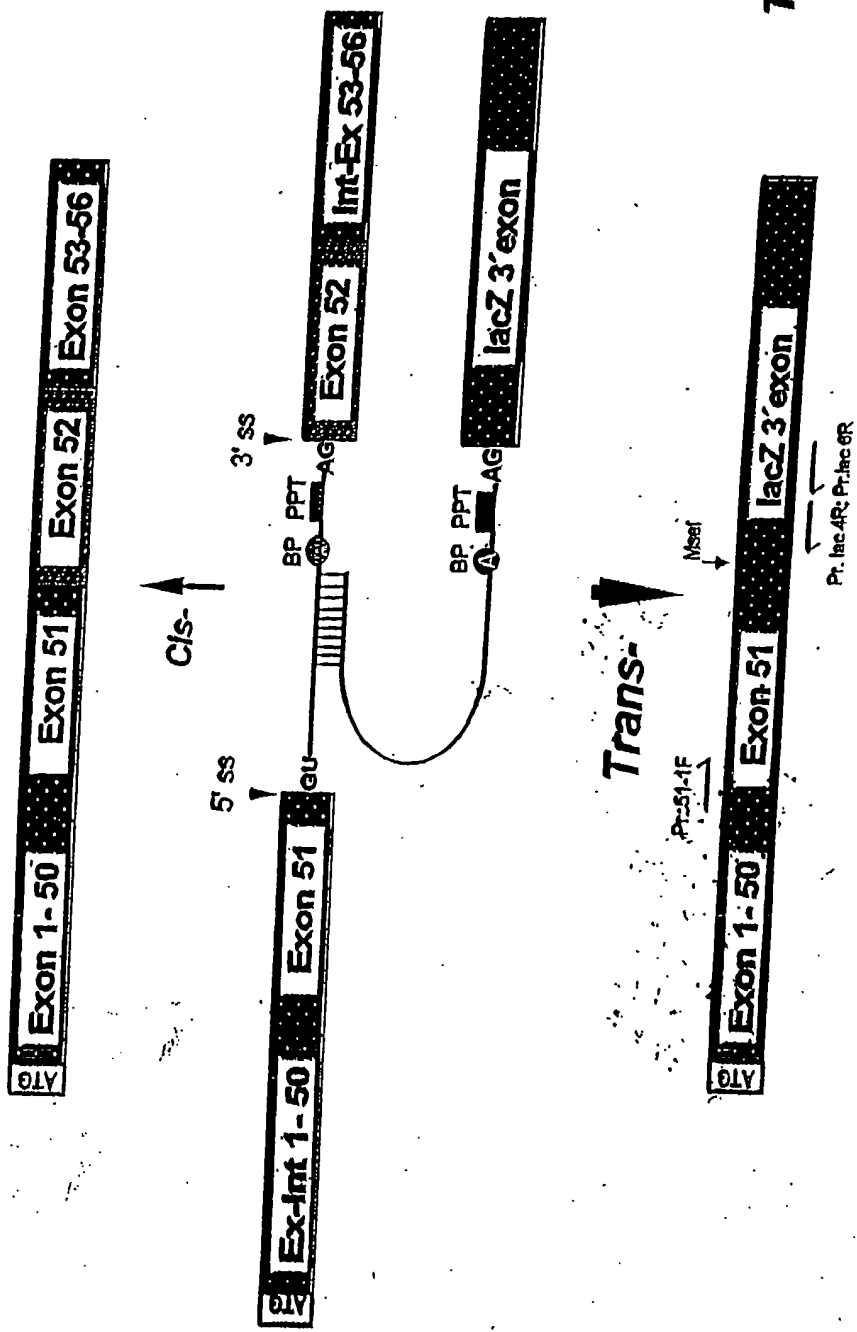
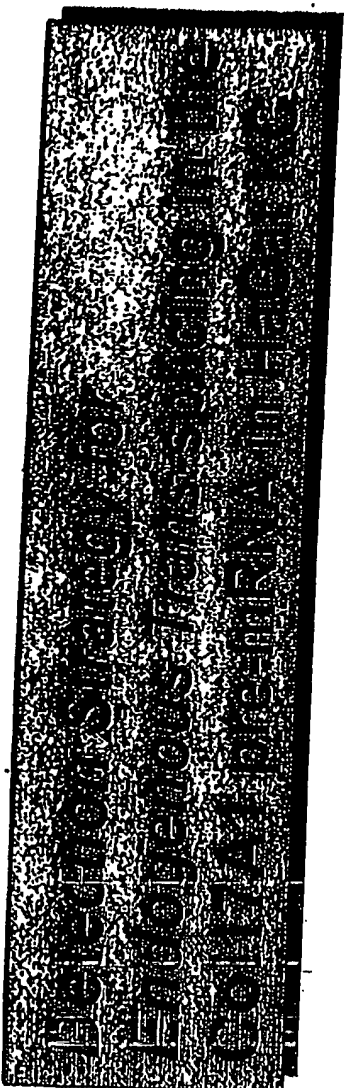
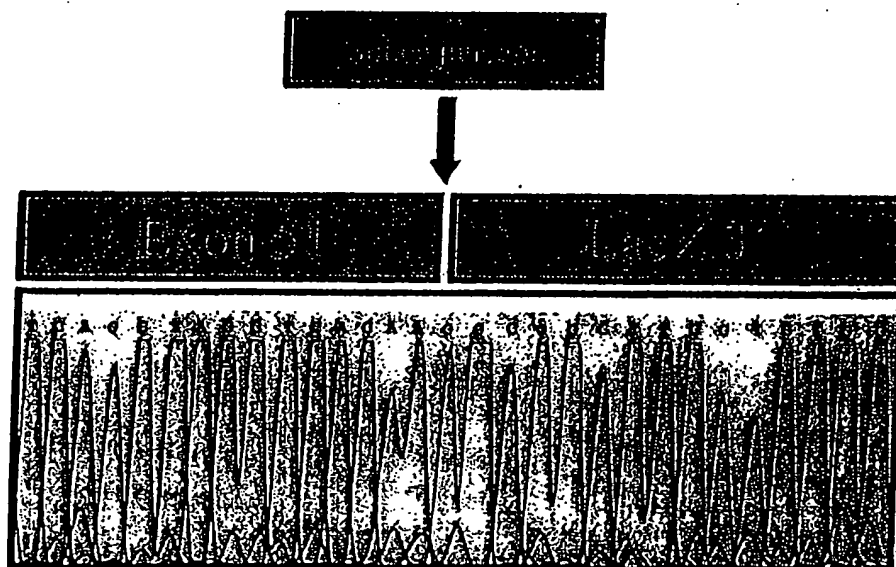


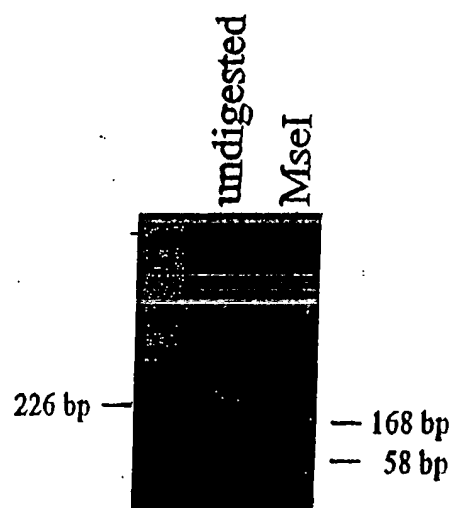
FIGURE 8

# Endogenous Transsplicing of Col17 pre-mRNA with PTM5 pre-mRNA

A)

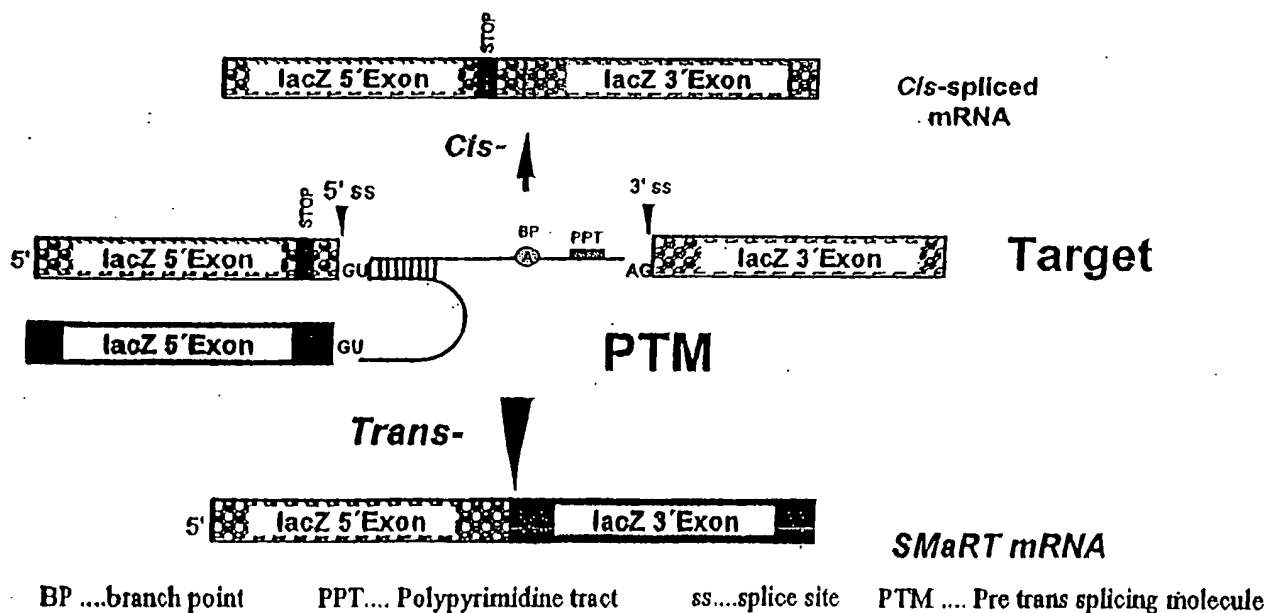


B)



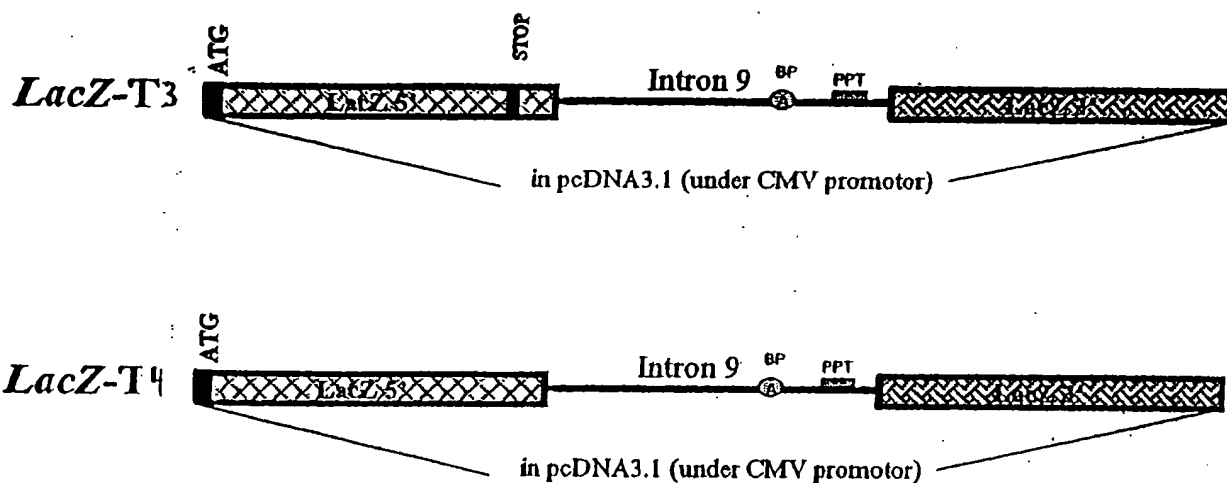
## FIGURE 9

### 5' trans-splicing in a LacZ-model system for hereditary diseases



## FIGURE 10

Target LacZ-T3 (containing Intron 9) and LacZ-T4 used for optimizing trans-splicing and transfection conditions

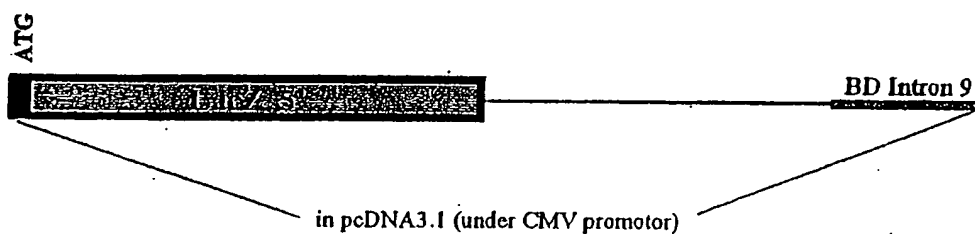




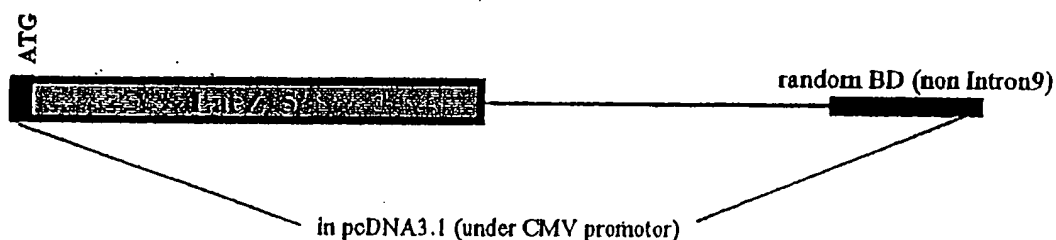
## FIGURE 11

LacZ-PTM-3 (Intron 9 specific binding domain) and LacZ-PTM-4 (non specific binding domain) for establishing optimal trans-splicing conditions

**Lac-PTM<sup>3</sup>**

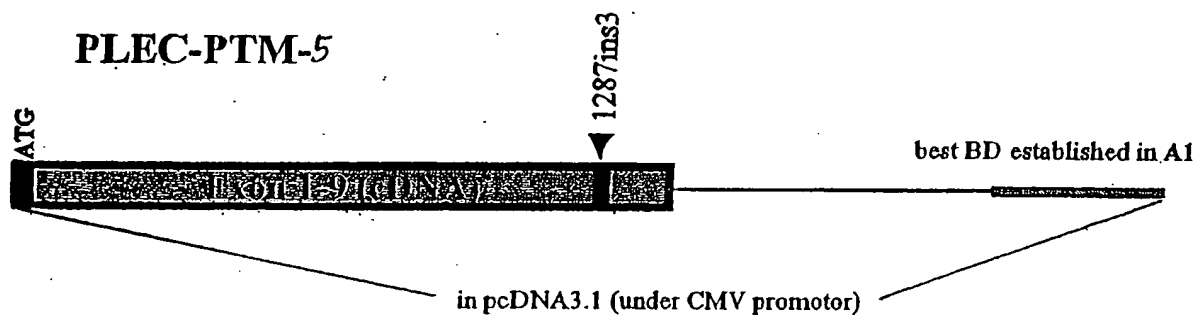


**Lac-PTM<sup>4</sup>**



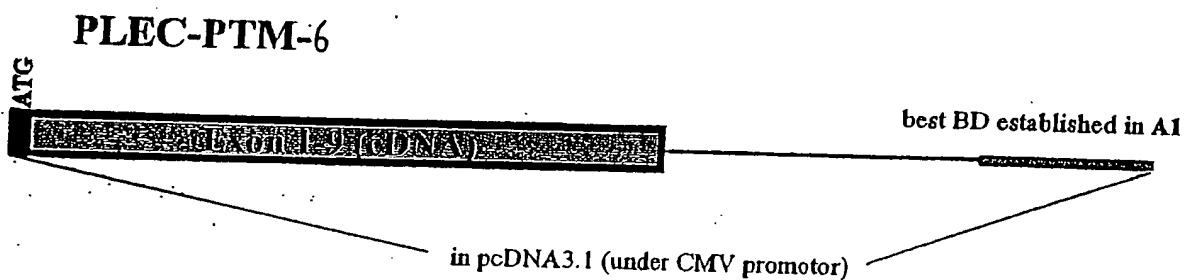
## FIGURE 12

PLEC-PTM-5 for the introduction of the 1287ins3 mutation  
in 293T cells



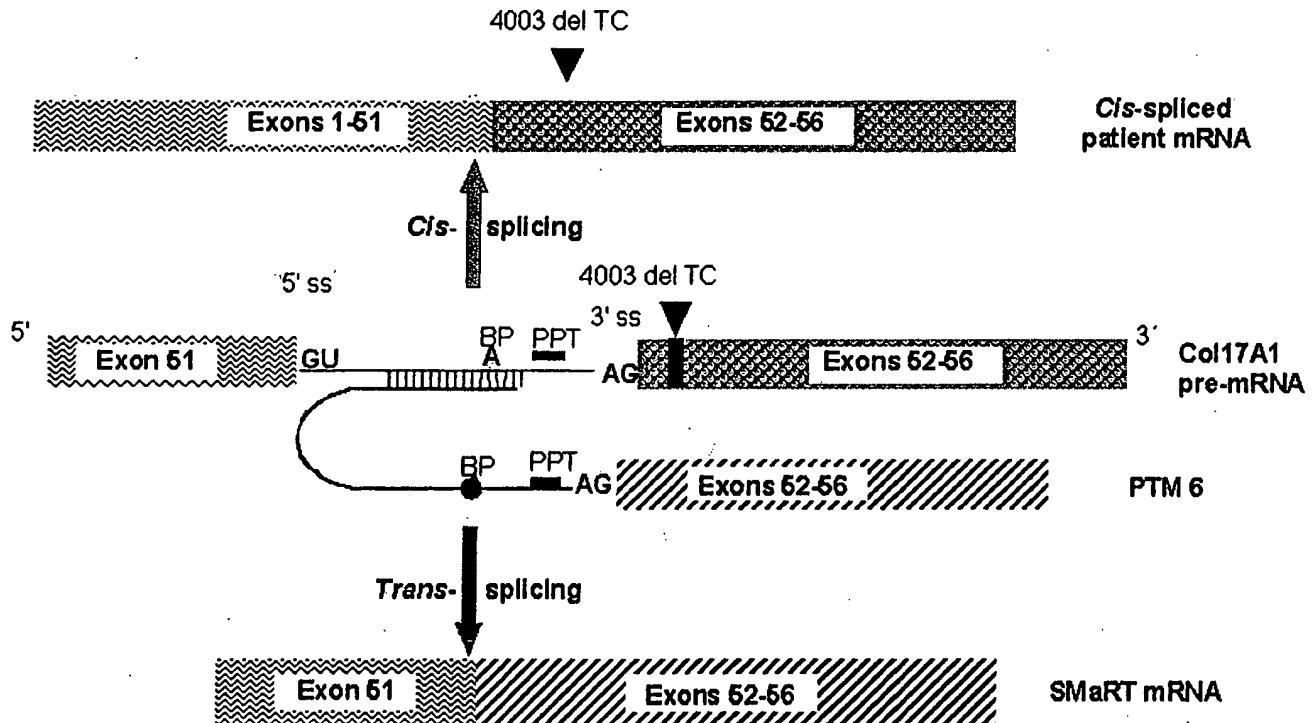
## FIGURE 13

PLEC-PTM-6 for the repair of the 1287ins3 mutation  
in plectin deficient patient cells



## FIGURE 14A

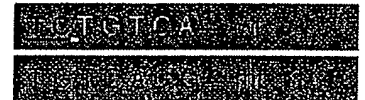
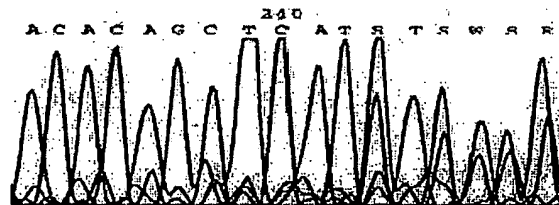
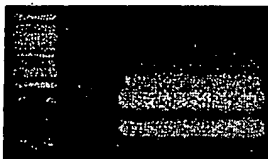
### **Trans-splicing Strategy for Col17A1 Gene (GABEB/generalized atrophic benign epidermolysis bullosa)**



## FIGURE 14B

# Semi-nested RT-PCR with BPAG2- primer 51-1F, 53-1R and 52-1R

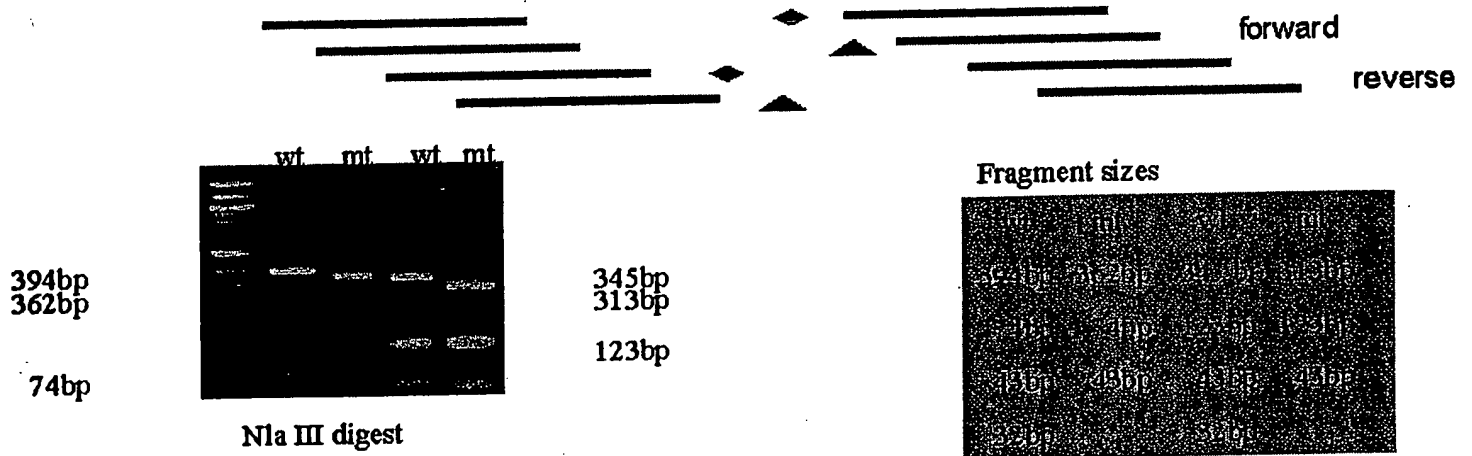
RT-PCR



F1  
B1  
C

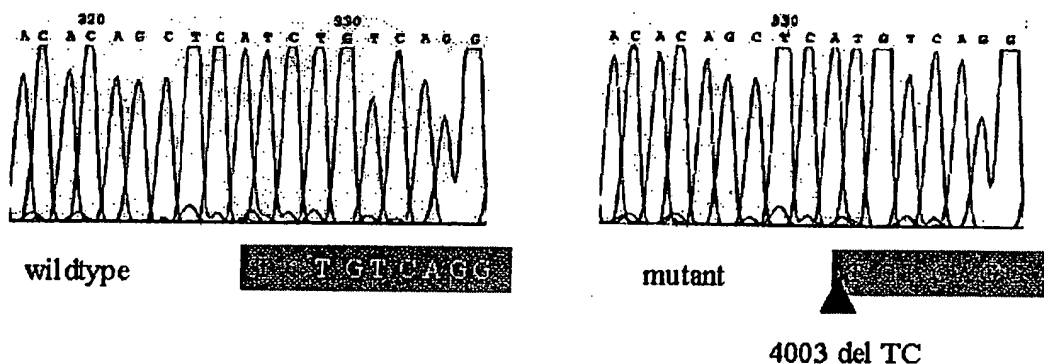
## FIGURE 14C

# TOPO Cloning + Nla III digest



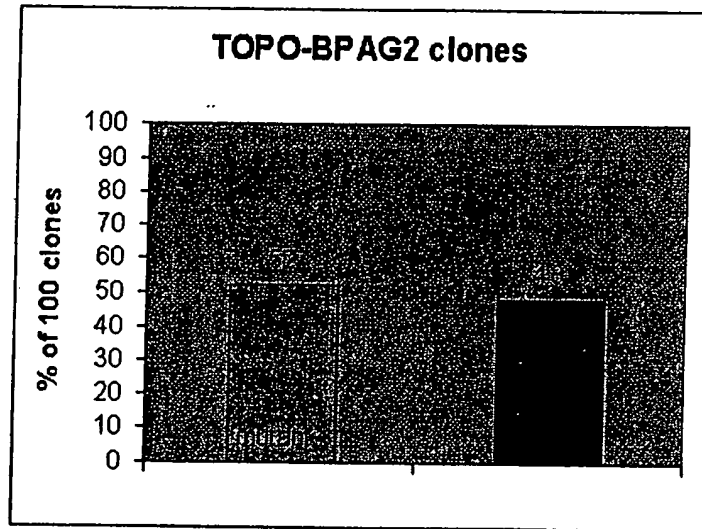
## FIGURE 14D

Sequencing of mutant and  
wildtype clones confirmed the  
correct *trans*-splicing of PTM6  
into GABEB cells



## FIGURE 14E

### Analysis of 100 clones

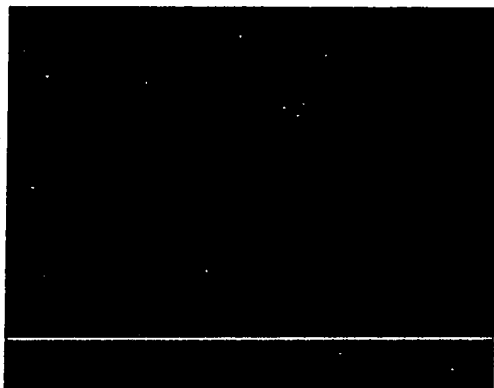




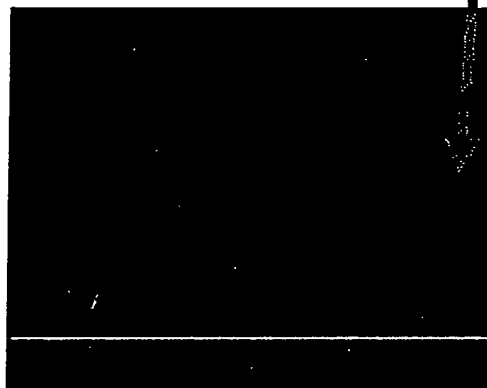
## FIGURE 15

# Immunofluorescence of PTM6- transfected GABEB cells

GABEB  
non-transfected

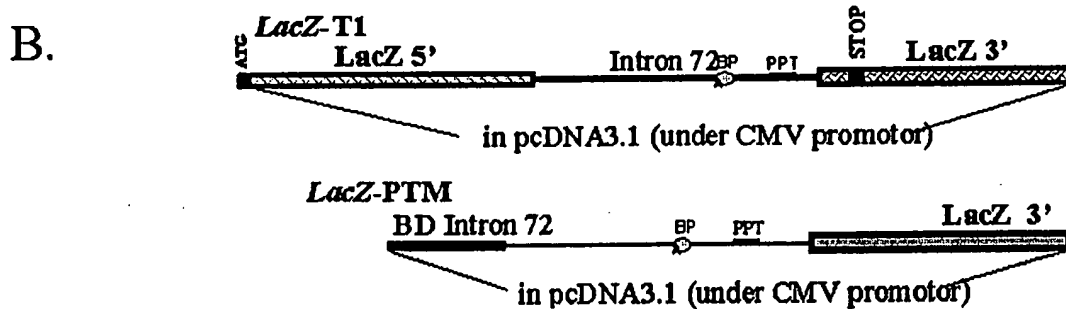
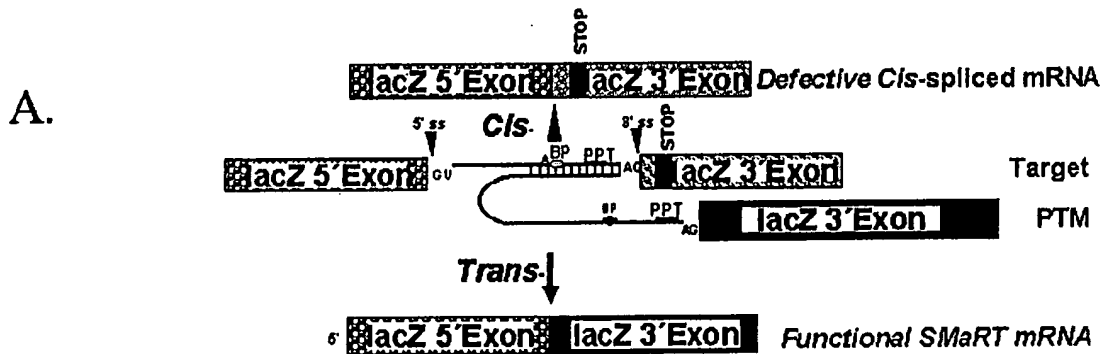


GABEB  
PTM6 transfected, 3 days



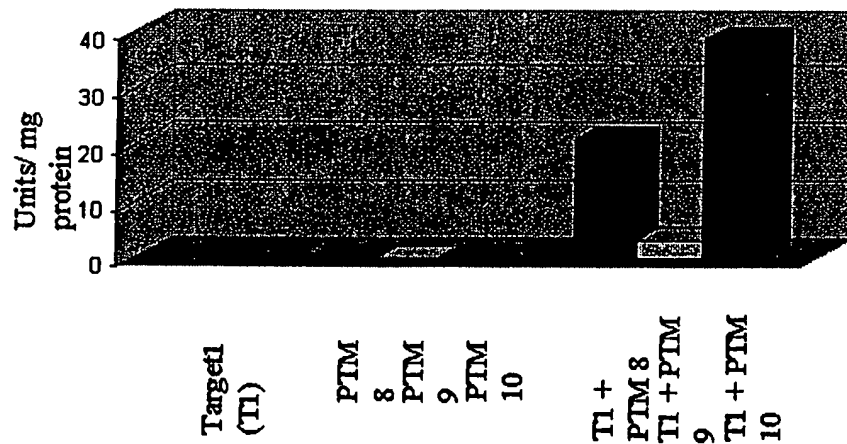
## FIGURE 16A-B

### *Trans*-splicing strategy for COL7A1 gene (Dystrophic epidermolysis bullosa)



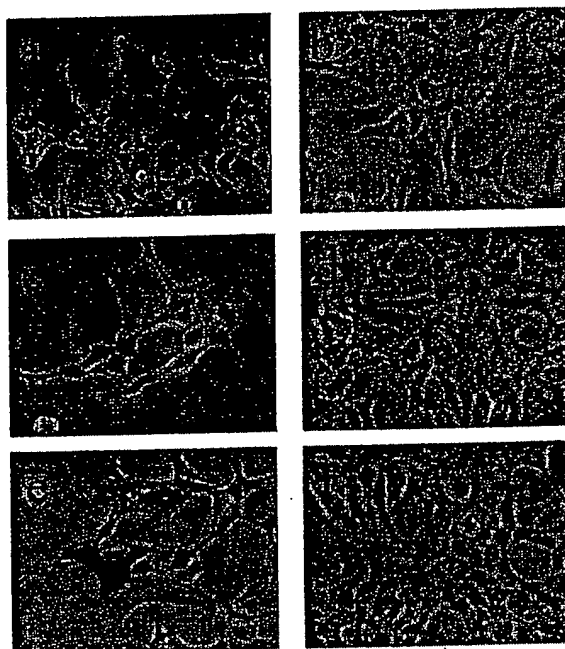
## FIGURE 17

# $\beta$ -gal Assay of a Cotransfection of Col7 Target T1 and PTM 8, 9, 10 in 293T cells



## FIGURE 18

# Beta-gal staining of Col7 PTM transfected human 293T cells



left panel: (a) Target + PTM8, (c) Target + PTM9, (e) Target + PTM10;  
right panel: corresponding control PTM without Target.